

CLAIMS

- 5 1. Use of NO, of a NO donor compound or of a compound able to release, promote or induce NO formation in cells, to prepare a medicinal product intended for the treatment or prevention of a disease resulting from deficiency of an adult gene in an individual through the re-expression of said homologous foetal gene.
- 10 2. Use of NO or of a NO donor compound or a compound able to release, promote or induce NO formation in cells according to claim 1, characterized in that said medicinal product is intended to reactivate the expression of at least one foetal gene in adult tissues such as to restore the presence and/or the localization of at least one foetal protein.
- 15 3. Use according to either of claims 1 or 2, characterized in that the foetal gene codes for the embryonic form of the protein encoded by the deficient gene.
- 20 4. Use according to any of claims 1 to 3, characterized in that the compound able to induce NO formation is L-arginine, or one of its derivatives, forming a substrate for NO-synthase or promoting availability of the substrate.
- 25 5. Use according to any of the preceding claims, characterized in that the definite gene is the dystrophin gene and the foetal gene is the utrophin gene.

6. Use according to any - of the preceding claims, characterized in that the deficient gene is the haemoglobin gene and the foetal gene is the foetal haemoglobin gene.

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7. Use according to any of the preceding claims, characterized in that the disease resulting from the deficiency of an adult gene is a muscular dystrophy, such as Duchenne or Becker muscular dystrophy, or thalassaemia or sickle-cell disease.

8. Pharmaceutical composition characterized in that it contains NO and/or at least one NO donor or a compound able to release, promote or induce NO formation in cells, associated in said composition with a pharmaceutically acceptable vehicle.

ASA
A2

Fig. 1

Fig. 1 (cont.)

Fig. 2A NXLT

Fig. 2A NXLT (cont.)

5 Fig. 2A NXLT (cont.)

Fig. 2B XLT

Fig. 2B XLT (cont.)

Fig. 2B XLT (cont.)

Fig. 3

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